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High-level factor VIII gene expression in vivo achieved by nonviral liver-specific gene therapy vectors.

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L3         14928 S (FACTOR IX) OR (F IX) OR (HFIX)
L4            14 S L1 AND L2 AND L3
L5            10 DUP REM L4 (4 DUPLICATES REMOVED)
L6            12 S L1 (S) L2
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L13           27 S L1 (P) PROMOTER (P) INTRON
L14           12 DUP REM L13 (15 DUPLICATES REMOVED)
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AU Miao C H; Ohashi K; Patijn G A; Meuse L; Ye X; Thompson A R; Kay M A

SO Molecular therapy : journal of the American Society of Gene Therapy, (2000 Jun) 1 (6) 522-32.
Journal code: 100890581. ISSN: 1525-0016.

TI Inclusion of the hepatic locus control region , an intron, and untranslated region increases and stabilizes hepatic factor IX gene expression in vivo but not in vitro.

AB We systematically compared human factor IX gene expression from a variety of plasmids containing different cis-regulatory sequences after transfection into different hepatocyte cell lines, or in vivo, after their injection into the livers of mice. Although there was a 1.5- to 2.0-fold variation in gene expression from cultured cells, a 65-fold variation was observed in the in vivo studies. We found that a plasmid containing the apolipoprotein E locus control region (HCR), human alpha1-antitrypsin (hAAT) promoter, hFIX minigene (hFIXmg) sequence including a portion of the first intron (intron A), 3'-untranslated region (3'-UTR), and a bovine growth hormone polyadenylation signal (bpA) produced the highest serum level of human factor IX, reaching 18 microg/ml (normal = 5 microg/ml) 1 day after injection. Although most of the plasmid DNAs resulted in transient gene expression, inclusion of an intron, a polyadenylation signal from either the 1.7-kb 3'-UTR or the 0.3-kb bpA, and the HCR resulted in persistent and therapeutic levels of hFIX gene expression, ranging from 0.5 to 2 microg/ml (10 to 40% of normal) for 225 days (length of experiment). These data underscore the importance of cis sequences for enhancing in vivo hepatic gene expression and reemphasize the lack of correlation of gene expression in tissue culture and in vivo studies.

AU Miao, Carol H. [Reprint author]; Thompson, Arthur R. [Reprint author]; Loeb, Keith R.; Ye, Xin [Reprint author]

SO Blood, (November 16, 2000) Vol. 96, No. 11 Part 1, pp. 210a. print.
Meeting Info.: 42nd Annual Meeting of the American Society of Hematology. San Francisco, California, USA. December 01-05, 2000. American Society of Hematology. FILE 'MEDLINE, EMBASE, BIOSIS, CAPLUS' ENTERED AT 15:50:06 ON 10 NOV 2005

CODEN: BLOOAW. ISSN: 0006-4971.

TI A nonviral approach: Long-term and therapeutic level human factor IX gene expression due to retention of optimal HFIX plasmids in hepatocytes after naked DNA transfer.

AB It was found that hFIX plasmids containing hepatic locus control region (ApoE-HCR), alpha1-antitrypsin promoter, hFIX cDNA, a portion of hFIX first intron, and a polyadenylation signal (from either bovine growth hormone or hFIX 3'-UTR) produced high level gene expression in mouse livers. Rapid tail vein injection of 20 mug plasmids in a large fluid volume produced 10mug/ml of hFIX protein (normal=5mug/ml) on the first day, which subsequently decreased to lower levels (Miao et al. (2000) Mol. Ther. 1, 522-532). Very interestingly, the plasma hFIX concentrations stabilized at 7-8 weeks in the range from 0.5 to 2 mug/ml (therapeutic for treating hemophilia B). These levels were maintained for over one year (duration of the experiments). Southern analyses showed that majority of the DNA were taken up by the liver. The mount of vector DNA retained in the cells

peaked 1 day post injection, then declined and stabilized at a constant level from mice infused by either high-expressing, or low-expressing plasmids. Restriction analyses showed that most of the vector DNA stayed in the same episomal forms as the original plasmid. RT-PCR analyses showed that the transcripts were only observed in the liver. The level of mRNA correlated with the protein expression overtime. Partial hepatectomy resulted in a significant decline in transgene expression, indicative of decreased episomal plasmid maintenance rather than transgene integration. Taken together, retention of the plasmids in the nucleus furnished the first step towards stable expression of the transgene, and the expression level were further controlled by the subsequent steps of transcription and processing to the stable transcript. Liver toxicity from the acute plasmid injection were evaluated by liver enzyme assays and histology. ALT and AST levels were raised 3-4 fold initially, then rapidly declined to normal levels at 3 to 10 days after injection, whereas serum billirubin levels remained normal at all times. Initial hepatic sections showed focal hemorrhage and necrosis representing less than 5% of the liver. Subsequent sections showed reparative changes resolving to histologically normal tissue with no significant fibrosis or inflammation. No significant differences were observed between plasmid injection and saline only control. These data established the foundation towards developing nonviral gene transfer strategy with optimal hFIX plasmids for the treatment of hemophilia B.

AU Miao, Carol H.; Thompson, Arthur R.; Loeb, Keith; Ye, Xin
SO Molecular Therapy (2001), 3(6), 947-957
CODEN: MTOHCK; ISSN: 1525-0016
TI Long-term and therapeutic-level hepatic gene expression of human factor IX after naked plasmid transfer in vivo
AB Naked DNA transfer of a high-expressing human factor IX (hFIX) plasmid yielded long-term (over 1 1/2 yr) and therapeutic-level (0.5-2 .mu.g/mL) gene expression of hFIX from mouse livers. The expression cassette contained a hepatic locus control region from the ApoE gene locus, an .alpha.1-antitrypsin promoter, hFIX cDNA, a portion of the hFIX first intron, and a bovine growth hormone polyadenylation signal. In contrast, a hFIX plasmid contg. the expression cassette without effective regulatory elements produced initially low-level gene expression that rapidly declined to undetectable levels. Southern analyses of the cellular DNA indicated that the majority of the input genome from either vector persisted as episomal forms of the original plasmids. Together with RT-PCR analyses of the transcripts, these data indicated that at least two processes are crit. for sustained gene expression: persistence of vector DNA and transcriptional/posttranscriptional activation. Liver regeneration after partial hepatectomy resulted in a significant decline in transgene expression, further suggestive of decreased episomal plasmid maintenance rather than transgene integration. Transaminase levels and liver histol. showed that rapid i.v. plasmid injection into mice induced transient focal acute liver damage (<5% of hepatocytes), which was rapidly repaired within 3 to 10 days and resulted thereafter in histol. normal tissue. No significant differences were obsd. between rapid injection of plasmid and saline control solns. Transient, very low level antibodies directed against hFIX did not prevent the circulation of therapeutic levels of the protein. Gene transfer of hFIX plasmid DNA into liver elicited neither transgene-specific cytotoxic effect nor long-term toxicity. These results demonstrate that long-term expression of hFIX can be achieved by nonviral plasmid transfer and suggest that this occurs independent of integration.

AU Miao, Carol H. [Reprint Author]; Ye, Xin; Thompson, Arthur R.
SO Human Gene Therapy, (September 20 2003) Vol. 14, No. 4, pp. 1297-1305. print.
ISSN: 1043-0342 (ISSN print).
TI High-level factor VIII gene expression in vivo achieved by nonviral liver-specific gene therapy vectors.
AB Two liver-specific nonviral gene transfer vectors have been developed to accommodate heterologous genes. The expression cassettes contain (1) a hepatic locus control region from the apolipoprotein E (ApoE) gene (HCR), (2) a liver-specific alpha1-antitrypsin promoter (HP), (3) a 1.4-kb truncated factor IX first intron (I) or a

synthetic minx intron (mI), (4) a multiple cloning site (MCS) for inserting cDNA sequences, and (5) a bovine growth hormone polyadenylation signal (bpA) to make pBS-HCRHPI-A or pBS-HCRHPmI-A. These vectors were first evaluated with reporter genes encoding human factor IX (hFIX) and green fluorescent protein (GFP). hFIX constructs, pBS-HCRHPI-FIXA and control pBS-HCRHP-FIXIA with the hFIX intron in its native position, produced comparable hFIX gene expression levels (0.5-5 mug/ml) 6 months after naked DNA transfer to mice, whereas the factor IX level from pBS-HCRHPmI-FIXA averaged about 50% lower. RT-PCR analysis of the mRNA indicated that introns inserted upstream from the cDNA were correctly processed and spliced. GFP expression was detected in 15-30% of the hepatocytes in pBS-HCRHPI-GFPA-treated mice. Next, a B domain-deleted human factor VIII (hFVIII) cDNA was inserted into the modified vectors. High-level hFVIII expression (up to 750 ng/ml) was achieved initially in both C57BL/6 mice and Rag2 mice. Moreover, therapeutic levels of hFVIII (20-310 ng/ml) circulated in Rag2 mice 6 months after treatment. These liver-specific gene expression cassettes can deliver a large, heterologous gene such as hFVIII cDNA to achieve high-level, persistent transgene expression after in vivo hepatic gene therapy.

IN Simonet, William S.; Lichenstein, Henry S.; Lyons, David E.
SO PCT Int. Appl., 75 pp.
CODEN: PIXXD2
TI Transgenic mammal with enhanced liver expression of a transgene using the human hepatocyte-specific enhancer element HCR
AB This invention provides a mammal with enhanced liver expression of a transgene. Also provided are: (1) a nucleic acid sequence useful in enhancing liver specific expression of a transgene, and (2) a vector contg. this nucleic acid sequence. The vector consists of a 774-bp portion of the human hepatocyte-specific control region (HCR, from vector pCI-CI'PX#8) linked a liver-specific promoter. Thus, HCR was operably linked to the human apolipoprotein E promoter and the ApoE intron 1 (including portions of the 5' and 3' exons), along with the SV40 polyadenylation sequence. The construct was linked to cDNA fragments encoding human interleukin-8, human keratinocyte growth factor, monocyte chemoattractant protein 1, or afamin, and microinjected into mouse embryos. The transgenic mice exhibited serum interleukin-8 levels of .gtoreq.100 ng/mL, whereas no interleukin-8 was detected in the serum of the nontransgenic mice. Circulating neutrophils levels exceeded 6000 units/.mu.L blood, whereas nontransgnic mice had a level of <1000/.mu.L blood.

IN Simonet, William Scott
SO U.S., 30 pp., Cont.-in-part of U.S. Ser. No. 141,322, abandoned.
CODEN: USXXAM
TI Tissue specific transgene expression by using a hepatocyte enhancer sequence
AB This invention provides a mammal with enhanced liver expression of a transgene. Also provided are 1: a nucleic acid sequence useful in enhancing liver specific expression of a transgene, and 2: a vector contg. this nucleic acid sequence. The invention further provides a non-human transgenic mammal contg. nucleic acid sequence comprising an HCR enhancer, the human ApoE promoter, the human ApoE intron 1 linked at its 5' end to the human ApoE exon 1 and at its 3'-end to a portion of the human ApoE exon 2, and at least a portion of the coding sequence of the transgene human IL-8, the transgene KGF, or the trans gene AFM.